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Section:	Prescription Drugs	**Effective Date:**	October 1, 2024
Subsection:	Neuromuscular Agents	**Original Policy Date:**	November 10, 2023
Subject:	Agamree	**Page:**	1 of 5

Last Review Date: September 6, 2024

Agamree

Description

Agamree (vamorolone)

Background
Agamree (vamorolone) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, Agamree acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which Agamree exerts its therapeutic effects in patients with DMD is unknown (1).

Regulatory Status
FDA-approved indication: Agamree is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older (1).

Agamree, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after Agamree withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events (1).

Agamree can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic. Corticosteroids reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection (1).

Agamree may affect cardiovascular and renal function, gastrointestinal perforation, behavior and mood, bone mineral density, and ophthalmic health. Blood pressure, serum potassium, signs of gastrointestinal perforation, mood, bone mineral density, and intraocular pressure should be monitored (1).

Agamree had lower rates of bone turnover versus prednisone. In clinical studies, there was a significant improvement in linear growth after crossover in the prednisone to Agamree group, and rapid reversal of prednisone-induced decline in bone turnover biomarkers in both crossover groups (2).

All immunizations should be administered according to immunization guidelines prior to starting Agamree. Live or live-attenuated vaccines should be administered at least 4 to 6 weeks prior to starting Agamree. Patients on Agamree may receive concurrent vaccinations, except for live or live-attenuated vaccines (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications (3-5).

Safety and effectiveness in pediatric patients below the age of 2 years have not been established (1).

Related policies
Amondys 45, Duvyzat, Elevidys, Emflaza, Exondys 51, Viltepso, Vyondys 53

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Agamree may be considered **medically necessary** if the conditions indicated below are met.

Agamree may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL of the following:

a. Genetic confirmation of DMD
b. Inadequate treatment response, intolerance, or contraindication to a ≥ 6 month trial of prednisone or prednisolone
c. Obtain a baseline motor milestone score from **ONE** the following assessments:
    i. 6-minute walk test (6MWT)
    ii. North Star Ambulatory Assessment (NSAA)
    iii. Motor Function Measure (MFM)
d. **NOT** given concurrently with live vaccinations
e. Absence of active infection [including tuberculosis and hepatitis B virus (HBV)]
f. If the patient has a history of hepatitis B (HBV) infection
    i. Prescriber agrees to monitor for HBV reactivation

Prior – Approval *Renewal* Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL of the following:

a. Stabilization **OR** improvement in motor milestone score from baseline from **ONE** the following assessments:
   i. 6-minute walk test (6MWT)
   ii. North Star ambulatory assessment (NSAA)
   iii. Motor Function Measure (MFM)
b. **NOT** given concurrently with live vaccinations
c. Absence of active infection [including tuberculosis and hepatitis B virus (HBV)]
d. If the patient has a history of hepatitis B (HBV) infection
   i. Prescriber agrees to monitor for HBV reactivation

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 6 months

Prior – Approval *Renewal* Limits

Duration 12 months

Rationale

Summary

Agamree (vamorolone) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Agamree acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The most common adverse reactions are changes in endocrine function, cardiovascular and renal function, gastrointestinal perforation, behavior and mood, bone mineral density, and ophthalmic health. Safety and effectiveness in patients less than 2 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Agamree while maintaining optimal therapeutic outcomes.

References

1. Agamree [package insert]. Coral Gables, FL: Catalyst Pharmaceuticals, Inc.; June 2024.
2. Guglieri M, Clemens PR, et al. Efficacy and Safety of Vamorolone vs Placebo and Prednisone Among Boys With Duchenne Muscular Dystrophy: A Randomized Clinical Trial. JAMA Neurol. 2022;79(10):1005-1014.
3. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 357–368.
4. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Endpoints in Duchenne Muscular Dystrophy: Longitudinal Natural History Observations Over 48 weeks from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 343–356.
5. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.

Policy History

Date	Action
November 2023	Addition to PA
March 2024	Annual review
June 2024	Annual review and reference update. Per SME, added description of safety outcomes from pivotal trial and updated t/f of prednisone to require a 6-month trial of prednisone or prednisolone
September 2024	Annual review and reference update. Per SME, changed initiation requirement wording to “genetic confirmation of DMD”

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 6, 2024 and is effective on October 1, 2024.